



4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-N-0967]

Prescription Drug User Fee Act Patient-Focused Drug Development; Announcement of Disease Areas for Meetings Conducted in Fiscal Years 2013-2015

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of Availability.

SUMMARY: The Food and Drug Administration (FDA) is announcing the selection of disease areas to be addressed during the first 3 years of Patient-Focused Drug Development. This 5-year initiative is being conducted to fulfill FDA's performance commitments made as part of the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). It provides a more systematic approach for the Agency to obtain patients' input on specific disease areas, including their perspectives on their condition, its impact on daily life, and available therapies. FDA selected these disease areas based on a set of selection criteria, the perspectives of the reviewing divisions at FDA, and the public input received on a preliminary set of disease areas published in the Federal Register on September 24, 2012.

ADDRESSES: The general schedule of fiscal years (FY) 2013-2015 meetings concerning Patient-Focused Drug Development, information on how stakeholders can prepare for them, and information on how stakeholders may leverage Patient-Focused Drug Development to generate

input on disease areas that are not addressed through the PDUFA V commitments can be found at the Web site for Patient-Focused Drug Development:

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>.

FOR FURTHER INFORMATION CONTACT:

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SUPPLEMENTARY INFORMATION:

I. Background

On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act (FDASIA) (Public Law 112-144). Title I of FDASIA reauthorizes the

Prescription Drug User Fee Act (PDUFA), which provides FDA with the necessary user fee resources to maintain an efficient review process for human drug and biologic products. The reauthorization of PDUFA includes performance goals and procedures that represent FDA's commitments during FY 2013-2017. These commitments are referred to in section 101 of FDASIA and are available on the FDA Web site at

<http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM270412.pdf>.

Section X of these commitments relates to enhancing benefit-risk assessment in regulatory decision-making. A key part of regulatory decision-making is establishing the context in which the particular decision is made. For purposes of drug marketing approval, this includes an understanding of the severity of the treated condition and the adequacy of the available therapies. Patients who live with a disease have a direct stake in the outcome of FDA's decisions and are in a unique position to contribute to the understanding of their disease.

FDA has committed to obtain the patient perspective on 20 disease areas during the course of PDUFA V. For each disease area, the Agency will conduct a public meeting to discuss the disease and its impact on patients' daily lives, the types of treatment benefit that matter most to patients, and patients' perspectives on the adequacy of available therapies. These meetings will include participation of FDA review divisions, the relevant patient community, and other interested stakeholders.

II. Disease Area Selection

On September 24, 2012, FDA published a Federal Register notice (77 FR 58849) that announced an opportunity for public comment on potential disease areas to be addressed throughout PDUFA V. In that notice, based on several criteria listed therein, FDA identified 39

disease areas as potential candidates for 20 public meetings and invited public comment on the preliminary list and on disease areas that were not listed. The Agency obtained public comment through a docket and a public meeting convened on October 25, 2012.

Almost 4,500 comments addressing over 90 disease areas were submitted by patients, patient advocates and advocacy groups, caregivers, healthcare providers, professional societies, scientific and academic experts, pharmaceutical companies, and others. The majority of comments were submitted by individual patients. The comments generally focused on one or more of the following: Nominations of support for individual disease areas or groups of disease areas, general suggestions for Patient-Focused Drug Development, and topics outside the scope of the program. Many comments discussed the impact of the disease on daily life and the symptoms that were most concerning to patients. Others addressed lack of treatment options or the nature of specific treatments. Over half of the comments received concerned lung cancer, narcolepsy, and interstitial lung disease. Other disease areas also received a significant number of comments, including migraine, pulmonary fibrosis, amyloidosis, myalgic encephalomyelitis/chronic fatigue syndrome, amyotrophic lateral sclerosis, chronic obstructive pulmonary disease, lysosomal storage disorders, peripheral neuropathy, dystonia, and fibromyalgia. Comments were received for numerous other disease areas not listed in this notice. Individual comments may be viewed at <http://www.regulations.gov/#!docketDetail;D=FDA-2012-N-0967>, or by visiting FDA Dockets Management at 5630 Fishers Lane, rm. 1061, HFA-305, Rockville, MD 20852.

Input from the public was particularly helpful for FDA in better understanding the aspects of diseases that are not formally measured in clinical trials as well as cases where available therapies do not directly impact the aspects of disease that matter most to patients. The extent of public comment for specific disease areas was one of many factors used to select the

disease areas for Patient-Focused Drug Development during FY 2013-2015. In selecting the disease areas of focus, FDA carefully considered the public comments received, the perspectives of reviewing divisions at FDA, and the following selection criteria, which were published in the September 24, 2012, Federal Register notice:

- Disease areas that are chronic, symptomatic, or affect functioning and activities of daily living;
- disease areas for which aspects of the disease are not formally captured in clinical trials; and
- disease areas for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels or functions.

FDA's selection also reflects the Agency's desire to include a diverse set of disease areas that represent the wide range of diseases the Agency encounters in its regulatory decision-making. These criteria, also published in the September 24, 2012, Federal Register notice, were overarching considerations that the Agency took into account in selecting the set of disease areas:

- Disease areas that reflect a range of severity, from diseases that are life-threatening to those that are mild and symptomatic;
- disease areas that have a severe impact on identifiable subpopulations, such as children or the elderly; and
- disease areas that represent a broad range in terms of size of the affected population, including common conditions experienced by large numbers of patients and rare diseases that affect much smaller patient populations.

Patient-Focused Drug Development was conceived as a mechanism to learn more from patients where their perspectives could be helpful to drug development and FDA's review of applications for new drugs in certain disease areas. For FDA's review divisions, this kind of input is most helpful when the impact of a disease on patients is not well understood or endpoints for studying drugs for a disease are not clearly defined or established. The potential to fill these information gaps by hearing from patients was also a key consideration in identifying the initial 12 disease areas.

FDA has selected the following diseases to be addressed in FY 2013-2015:

- Alpha-1 antitrypsin deficiency;
- breast cancer;
- chronic Chagas disease;
- female sexual dysfunction;
- fibromyalgia;
- hemophilia A, hemophilia B, von Willebrand disease, and other heritable bleeding disorders;
- HIV;
- idiopathic pulmonary fibrosis ;
- irritable bowel syndrome, gastroparesis, and gastroesophageal reflux disease with persistent regurgitation symptoms on proton-pump inhibitors;
- lung cancer;
- myalgic encephalomyelitis/chronic fatigue syndrome;
- narcolepsy;
- neurological manifestations of inborn errors of metabolism;

- Parkinson's disease and Huntington's disease;
- pulmonary arterial hypertension; and
- sickle cell disease.

A schedule of the meetings planned for each year can be found at the FDA Patient-Focused Drug Development Web site described in the following section of this notice.

FDA will initiate a second public process to determine the list of disease areas for FY 2016-2017. The Agency recognizes that there are many more disease areas than can be addressed in the planned FDA meetings under PDUFA V, and FDA will seek other opportunities to gather public input on disease areas not addressed through this PDUFA V commitment. FDA also encourages stakeholders to identify and organize patient-focused collaborations to generate public input on other disease areas with regard to the types of questions addressed through this PDUFA commitment, using the process established through Patient-Focused Drug Development as a model. More information on other opportunities for gathering patient input can be found on the Patient-Focused Drug Development Web site.

III. Patient-Focused Drug Development Web Site

FDA has a Web site on Patient-Focused Drug Development:

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>. This Web site contains the general schedule of upcoming meetings for FY 2013-2015, information on how stakeholders can prepare for upcoming meetings, and information on how stakeholders may leverage Patient-Focused Drug Development to generate input on disease areas not addressed through the Patient-Focused Drug Development PDUFA V commitment. The Web site will be updated as new information becomes available.

Dated: April 5, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

[FR Doc. 2013-08441 Filed 04/10/2013 at 8:45 am; Publication Date: 04/11/2013]